



Annual Report and Financial Statements

To the year ended
31 December 2021

myotubular trust
FINDING STRENGTH



UK Registered Charity, 1137177
A Company Limited By Guarantee

TRUSTEES' ANNUAL REPORT 31 December 2021

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The Trustees of the Myotubular Trust take pleasure in presenting their annual report and the financial statements for the year ended 31 December 2021.

Objects and Activities

The objects of the Myotubular Trust as agreed by the Charity Commission are;

- the relief of disability and the extension of life for babies, children and adults suffering from myotubular myopathy,
- by promoting the study of, and research into, the treatment and cure of the muscle weakness caused by myotubular myopathy.

The Trust focuses on five outcomes to enable the achievement of these objectives:

1. Bringing families together
2. Promoting and funding international research
3. Sharing knowledge
4. Promoting innovative thinking and collaboration
5. Working with our extended families.

We bring about the public benefit required of all charities, by the activities of:

- making grants to organisations and individuals.
- providing support, advice, and information.
- sponsoring research.

Myotubular Myopathy and Centronuclear Myopathy

Myotubular myopathy is a rare and extremely life-threatening muscle condition, which causes profound muscle weakness, usually from birth. It is one of a group of conditions called centronuclear myopathies – called this because under a microscope the nucleus of the muscle cell of someone affected is in the centre, rather than at the edge of the cell.

These conditions are genetic in inheritance. The gene mutation for x-linked myotubular myopathy is carried on the x chromosome (xlmtm), and is the most common form, affecting (mainly) boys. They usually do not survive their babyhood – the statistics have been under review in the last few years and the current view is that 50-75% die in their first year, and very few make it to adulthood.

Even rarer genetic forms, either dominant or recessive in inheritance, can affect both males and females and are generally milder in severity. The terms myotubular and centronuclear myopathy both describe 'our' condition.

Children and adults with myotubular myopathy usually require vigilant nursing care, ventilators to breathe, power wheelchairs to get around, and are often fed via a tube into the stomach. Carrier females, and those affected by some of the rarer forms which can manifest in adult life, may develop muscle weakness and problems with eating and drinking; walking and movement; muscle fatigue and susceptibility to respiratory weakness and associated infections.

In 2021, a young boy who was taking part in the gene therapy trial for x-linked myotubular myopathy sadly died. We would like to take this opportunity to extend the deepest sympathies of the Myotubular Trust to his family and those who loved and took care of him. This was the fourth death in the gene therapy trial. As a result the trial has been put on hold by the FDA.

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Taking stock in 2021

February 2021 marked 15 years since the founding of the Myotubular Trust. The year 2021 also marked 25 years since the laboratory of Jocelyn Laporte in Strasbourg discovered the *mtm1* gene. In the 25 years since then, and the 15 years since the Trust was founded, substantial progress has been made in understanding myotubular and centronuclear myopathy. Research into a treatment, or cure, has made great steps forward; the first clinical trials in humans have begun; and there have been life enhancing and life lengthening improvements in supportive care for those living with myotubular and centronuclear myopathy.

However there remain many questions about the condition, and why and how these genetic mutations, and the proteins they impact, cause muscle weakness. There have been deeply disappointing setbacks in the clinical trials, particularly the gene therapy trial, which is now on hold after the death of 4 participating patients. Young boys also continue to die from the complications caused by myotubular myopathy, and for our community 2021 was a particularly tragic year of losses, leaving behind many heartbroken families.

The Trust was established in 2006 by two parents of boys with myotubular myopathy in order to raise research funds. There was no other way for passionate parents to ring-fence money to fund research into a treatment or cure for this condition, even though we did at the time approach some larger charities for whom we had hoped to fundraise. We also realised very quickly that it was essential to support the profile of the condition within the neuromuscular research community – to compete for brains and innovative ideas – and to support those living with, and bereaved by, myotubular myopathy.

In 2021, after a decade and a half, we took stock of where the Myotubular Trust was at, and although we take great comfort in knowing that we, with the support of so many families, have made a real and substantial difference to progress in finding a cure or treatment for this condition, it is very clear that there remains a lot more to be done. There are new understandings of the condition particularly emerging from the clinical trials, which present a number of new challenges, but also new avenues of research. We need to be here to voice the urgency of this work, and to be a source of funding too. We know that after these two incredibly difficult years it is now more important than ever to keep myotubular myopathy at the forefront of groundbreaking research.

We are dedicated to continuing to make a meaningful difference - there is clearly a vital role for a disease specific research funder in the world of rare disease, along with a tangible need to support patients and families. We are conscious though of 'Founder syndrome' and the voice of the living patient. We aim to take every opportunity to invite in other patients, parents and families to take key roles and become leading voices in this work. We recognise that no one organisation can provide all the answers, and it is our ambition in the next 5 years to support the initiatives of others, particularly our younger community.

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Since founding in 2006...we have:

1. Raised over **£2.5M**, through family-led fundraising, networks and relationships.
2. Made **16 research grants** to researchers in world class laboratories, with the support of a highly regarded Scientific Advisory Board.
3. Funded the proof of principle project for gene therapy that led to the **first ever clinical trial** for myotubular myopathy.
4. Funded **other proof of principle projects** that are in clinical trial.
5. Funded research grants that contributed to the discovery of **two new genes** implicated in centronuclear and myotubular myopathy.
6. Hosted **five (in person) family conferences**, bringing together affected individuals and families, researchers, clinicians, and other health professionals, and arranged a number of family meetings by **Zoom during the pandemic with global experts and scientists**.
7. Created the first disease specific international **Patient Registry** to gather vital information on the condition and funded its migration to the stability and credibility of a university setting.
8. Sponsored a **Standards of Care** meeting for clinicians, patients, and their healthcare professionals at the Royal Brompton Hospital.
9. Participated in a European Neuromuscular Centre workshop on Mouthpiece Ventilation, resulting in the publication of **MPV international best practice guidelines**.
10. Created a great band of **committed supporters**, raising funds for us year on year.
11. Developed strategic relationships with **key stakeholders** in the fields of neuromuscular disease, rare disease, UK regulatory bodies, and highly regarded UK children's charities.
12. And *most importantly*, brought together **families** affected by myotubular and centronuclear myopathy, who support the Trust's fundraising and each other's lives.

In 2021 specifically...we have:

1. Raised nearly £150,000.
2. Invited applications for our 12th research grant round.
3. Organised and hosted a Scientific Update webinar for patients and families, with leading experts presenting on clinical trial updates, liver issues, drug repurposing and Covid-19.
4. Organised and hosted a webinar for the UK patient community on 'Coping through Covid and beyond', delivered by a counselling psychologist from the Royal Brompton Hospital, London.
5. Presented at the symposium 'Turning the Tide – respiratory management of neuromuscular patients in the era of new treatments'.
6. Partnered with the UK regulator, the MHRA, to do a joint presentation on '*Patients as Partners in Medicines Development*' at the BioIndustry Association – MHRA Regulatory Innovation Conference.
7. Presented for LifeArc on drug repurposing for rare diseases and contributed to their published expert advice.
8. Developed, in collaboration with University College Cork, a HomeCare survey to gather information on home care packages.
9. Collaborated with the US patient organisation, MTM-CNM Family Connection, to set up a condition specific Liver Discussion Workgroup, bringing together a range of leading experts, experienced clinicians, and biotechs to fast-track cooperation on the understanding of liver issues emerging from current clinical trials.
10. Contributed to the first ever Congenital Myopathies Masterclass for European healthcare providers. The Masterclass was organised by TREAT-NMD and included sessions led by world leading experts on myotubular and centronuclear myopathy.
11. For the 15th year since founding, we have raised 100% of our running costs separately from the funds raised by families and supporters, meaning that once again we meet our ambition to dedicate all the funds raised by supporters into research.

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Family Support in 2021

Advocacy and Family Support

We continued, in 2021, to provide by phone and conference call facility, the support we provided in person pre-pandemic. We also ensured that the most up to date information on Covid 19 and vaccinations for those living with NMD was available for families, and we collaborated with the UK muscle disease charity, MDUK, to channel specific questions from patients directly to relevant experts.

We learnt very early on in the Trust's 15 years that specific support is needed for myotubular and centronuclear myopathy patients and families. This is, in the main, because centronuclear and myotubular myopathy are so rare that usually the child's or patient's GP or doctor will never have come across the condition before - making being diagnosed a frightening and bewildering experience for all concerned. Being hurled suddenly, and usually without warning, into a world of medical jargon, high level nursing, complex healthcare and social care systems, families and individuals are grateful to know they are not alone, and that we, and others like them, are willing to share the burden of responsibility by helping them understand their experiences and to navigate the systems.

At a time when parents are supposed to be celebrating the birth of an infant, our families are often instead in the position of having to make important choices around giving their child the best quality of life, and a chance of longer-term survival. The information these families are given to make those decisions is sometimes inaccurate, often patchy, and even delivered by someone who has never seen a child with the condition before.

When a baby is finally stable enough to go home, it is not unusual for the transition process from hospital to home to take 1 year to 18 months. There are huge differences between the capability of different hospitals, different clinical commissioning groups, and different community nursing support services in making this happen in a timely fashion. This has been further complicated since the pandemic began given the challenges of bringing together the multi-disciplinary teams necessary for this complex step, and the high demand for respiratory and ventilatory trained agency staff.

Therefore, in 2021, we developed a survey, in collaboration with University College Cork, to gather reliable, objective, analysable data on home care packages in place for myotubular and centronuclear myopathy patients throughout the UK. With that information we hope to be able to support new families and their healthcare teams at a very difficult time.

The need caused by a lack of understanding of the impacts of such a rare, devastatingly life threatening condition re-emerges many times throughout a patient's life - and more often at times of change, such as when there is a physical change (such as losing the ability to walk or scoliosis), during periods of ill health, when moving home or into a new phase of education or work, around family planning, or practical day-to-day living issues. So often experiences and information signposting are needed again and again by families. In 2021 we have built up a substantial repository of publicly available patient case studies and experiences to be shared. We are very grateful to all the patients and families who have contributed to these case studies. They are invaluable.

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Research in 2021

The Myotubular Trust was set up to raise funds to leverage the research findings being made, and techniques being trialled, in 'more common' muscle diseases. The ultimate goal of the Trust is to invest in a diverse number of routes to a cure for all genetic forms of myotubular and centronuclear myopathy, or in treatments that alleviate the most severe symptoms. This we do by investing in the very best translational research, peer reviewed to the highest international standards, supported by an eminent Scientific Advisory Board.

Grants in progress

Tamoxifen clinical trial

This grant, which is co-funded with Great Ormond Street Hospital Children's Charity, is for the UK arm of a clinical trial to test how well the well-known oncology drug, Tamoxifen, works in improving motor and respiratory function in patients with myotubular myopathy. The trial is being led by Dr Giovanni Baranello, and Professor Francesco Muntoni, at the UCL Great Ormond Street Institute of Child Health. It will involve children in the UK who are affected by x-linked myotubular myopathy, and is part of the global drug repurposing trial that resulted from the work the Trust funded with Dr Jim Dowling at Sick Kids Canada. The global trial is led by Dr Dowling.

The mTORC pathway

Dr Karim Hnia at the world-renowned French institute, INSERM, with 2-year Myotubular Trust funding is investigating the question "Is the mTORC1 pathway a route to treatment in x-linked myotubular myopathy?"

mTORC1 is a master regulator of muscle growth – from the early steps of muscle cell differentiation in the womb, to the work of muscles in adult life. The work of Dr Hnia's laboratory has shown that mTORC1 is 'overactivated' when XLMTM is present. What makes this a very interesting project (apart from the highly positive peer review) is that there are a number of drugs already licensed to regulate mTORC1 levels for other diseases.

Clinical trial update

Gene therapy

Virus mediated gene therapy received funding from the Myotubular Trust as far back as our first grant award in 2009 to Dr Ana Buj Bello at Genethon, Paris. Dr Buj Bello established proof of principle for this gene therapy, and with the success of her research funded by the Trust (grants in 2009 and 2012), other funders subsequently joined in too. *"It is the result of perseverance and joined forces. Thanks a lot for your support all along these years, your contribution has been crucial, you can be proud of it"* Dr Buj Bello

Dosing patients in this trial began in 2018, and there have been some remarkable outcomes for a number of patients. Ventilator independence has been achieved by boys previously on 24 hour ventilation and generally 'dramatic clinical improvements' were seen, as noted in the academic literature. Essentially, what has been proven is that gene therapy can reverse the severest of symptoms of myotubular myopathy.

Tragically though, 4 young patients died during the trial and it is now on hold while the FDA and the sponsoring company investigate thoroughly. What has become very clear though is that there are aspects of the condition that we do not fully understand yet – including in relation to the liver.

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Antisense Therapy

The 3 year grant, “*Reducing DNM2 as a novel therapeutic target for CNM*”, made by the Myotubular Trust to Jocelyn Laporte’s team at the laboratory IGBMC in 2016 “*validated a deliverable approach*”, and formed a major contribution to the pre-clinical studies preparing the way for a clinical trial to test this approach in humans—who have either the x-linked MTM or Dynamin 2 form of the condition.

In 2019, Dynacure, a start-up biotech based in Strasbourg, secured approval for a clinical trial for those affected by the MTM1 and DNM2 genes, and this trial was due to begin in 2020. One patient began treatment, but unfortunately the pandemic put the trial on pause for a number of months. However, by the end of 2020 recruitment was restored, and initial dosing began in several countries in Europe, including in the UK. This progressed throughout 2021.

Drug repurposing

2020 was the third year of our 3-year drug repurposing research grant to Dr James Dowling at the Hospital for Sick Children, Toronto, Canada. One of the aims of this grant was to determine the optimum dosage of Tamoxifen to take to clinical trial. By the end of 2020 this work had progressed successfully enough for clinical trial planning to be well advanced in sites in Canada, the US, and the UK (see grant announcement below).

Drug repurposing has the benefit of bringing drugs that have already been safely taken by thousands, maybe millions of other people, to a new group of patients. As safety has already been established, the length of clinical trial required to test such a drug for a new patient group, and the proof required for the regulatory authorities, can be much reduced. Patients, particularly those already living with a high degree of vulnerability, may also feel a lot more confidence and faith in a known drug.

On the road to all these clinical trials, the role of the Trust was to fund the proof of principle/validation work being proposed by these outstanding researchers, paving the way for companies like Astellas Gene Therapies and Dynacure, or academic institutions such as Sick Kids Toronto and Great Ormond St. London, to take them to clinical trial. That funding makes a fundamental difference, and we are immensely grateful to all our supporters who have made this possible.

We are also immensely grateful to our Scientific Advisory Board (SAB) for their time and their highly impressive expertise and understanding. The calibre of applications the Myotubular Trust attracts is due in no small part to the reputation of this group of scientists.

The academic members of the SAB are:

- Professor Francesco Muntoni, Head of the Dubowitz Neuromuscular Centre, Great Ormond Street Hospital
- Dr Meriel McEntagart, Consultant Clinical Geneticist, St. George's Hospital, London
- Professor Michael Duchon, Professor of Physiology, University College London
- Dr Susan C Brown, previously Reader in Translational Medicine, Department of Veterinary Basic Science, Royal Veterinary College, University of London
- Professor Volker Straub, Harold Macmillan Professor of Medicine, Consultant in Neuromuscular Genetics and Paediatrics, Newcastle University
- Professor Dominic Wells, Professor in Translational Medicine, Royal Veterinary College

We are indebted for the guidance they gave to the Myotubular Trust throughout the year. We are also very grateful to the lay members of our SAB for their time. The lay members include parents, bereaved parents and patients.

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Financial Review

Sustainable Fundraising – the Highlights of 2021

In 2021 the Trustees were gratified that we were able to raise £144,770, despite pandemic related challenges still fundamentally impacting charity fundraising. Our continued revenue stream is testament to the strength of support that a small number of affected families enjoy in their communities – the 'ripple effect' from each child into an extended network of supporters has been so critical to our growth and stability.

Despite the year that was in it, our families were able to organise the now famous Jack Blunsdon annual golf tournament – in its 15th year! – and Monte's Golf Cup had its inaugural event. Young Rian organised his own 100K Wheel sponsored event, and many other families took part in virtual fundraisers and sponsored events such as the 2021 virtual 10K.

Sadly, 2021 was a year of many losses and we extend our gratitude to the families and friends of those boys who in their time of grief considered the Myotubular Trust for donations.

We were fortunate to be supported by Jeans 4 Genes and the Edward Gosling Foundation, as well as Global's Make Some Noise and IGY Foundation continued support. We thank them all for their financial and moral support. Thank you for seeing the value of the work we do.

Once again we had an increase in standing orders during 2021, probably as people realised how difficult it was for charities during social distancing and lockdowns. Thank you to you all.

Fundraising Practice

The Trustees take their responsibilities to fundraising very seriously. To that end, we ensure that we adhere to the Fundraising Regulator's code of best practice and meet the Charity Commission's six principles of charity fundraising. For us, at our size, the areas of best practice we pay attention to are;

- providing information on how to fundraise safely,
- considered communication with all fundraisers, which is appropriate to our strategy,
- carrying out a proportionate process of due diligence around fund raising proposals,
- making a record of the issue and return of any charity collection materials,
- securing cash donations and banking them as soon as possible,
- not sharing personal data without explicit consent,
- including opt-out information on fundraising communications sent to a named individual,
- processing unsubscribe requests in a timely way,
- using funds as they have been directed by specific donors, in particular allocating restricted funds appropriately.

We adhere to the Data Protection Act 2018, General Data Protection Regulation (GDPR) and pay data protection fees to the Information Commissioner's Office (ICO).

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Reserves Policy

The Trustees sole aim in expending cash reserves is to fund research into a cure or treatment for myotubular myopathy and they do not consider it appropriate to hold long-term reserves.

However, as each research project is granted, those funds will be held available to be released in staged payments, and new awards will only be made if the full cost of the relevant research project is available, and available to be ringfenced. This prudent financial policy is appropriate given our size and specialism.

This policy meant that instead of being hit by a cash flow crisis in 2020 and 2021, and the even worse possibility of not being able to pay for committed research grants, we were even able to announce new grant awards in 2020, announce another grant call for 2021 and prepare to announce another call in 2022.

Plans for future periods

The Trust aims in 2022 to:

- Maintain excellence in family support, advocacy, information provision, and continue to make the introductions that families tell us are invaluable.
- Launch the Care Hours Survey as an academically led study with full ethical approval.
- Announce our 13th grant call.
- Maintain the impetus behind the Liver Discussion WorkGroup and seek ways to influence the vital progression of work to understand the implications of liver issues in myotubular myopathy.
- Continue to work with key stakeholders in the neuromuscular disease community, and strategic rare disease organisations.
- Continue to work with regulatory authorities – particularly the MHRA in the UK - on behalf of patients with myotubular and centronuclear myopathy.
- Plan ahead and prepare effectively for any future NICE review of treatments for myotubular and centronuclear myopathy, facilitating families' and patients' input.
- Partner with and support TREAT-NMD on the long-term excellence and sustainability of the Myotubular and Centronuclear Myopathy Patient Registry.
- Continue to share information with, and promote collaboration between, other family and patient organisations, relevant scientific interest groups and research organisations.
- Continue to secure separate funding to cover the Trust's running costs.
- Maintain our 2021 support of fundraising events, despite the continued challenges brought about by the pandemic.

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Reference and administrative details of the Charity, its Trustees and advisors

The following were Trustees of the Trust and held office during the above period:

Patricia Allen
Gary Browning
Greg Fowler
Andrew Lennox
Anne Lennox
James Rosling

Trustees are appointed by the board of trustees and serve for three years (five years for Anne Lennox) after which period they may put themselves forward for re-appointment. The trustees meet three times per annum.

Office

Myotubular Trust

- charity registration number 1137177
- company registration number 07260229

15a Barnard Road
London SW11 1QT

Bankers

CAF Bank Limited
Kings Hill
West Mailing
Kent
ME19 4JG

The Co-operative Bank
PO Box 250
Skelmersdale
WN8 6WT

Independent Examiner

Michael Stone MA ACA
Jamieson Stone LLP
Windsor House
40/41 Great Castle Street
London
W1W 8LU

Structure, Governance and Management

Constitution

The Myotubular Trust is an incorporated charity limited by guarantee and its governing document is the memorandum and articles of association dated 21 May 2010. The memorandum and articles of association has the same objects as that of the Trust Deed dated 26 January 2006 of the unincorporated charity. The Trust obtained charitable status under Section 4 of the Charities Act 1960 from the Charity Commission on 19 April 2006 under registration number 1113809 and following incorporation on the 20th May 2010 was registered with the Charity Commission with registration number 1137177, and with Companies House with company registration number 7260229.

Organisation and the Trustees

In selecting individuals for appointment, the trustees will have regard to the skills, knowledge and experience needed for the effective administration of the charity.

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Trustees' responsibilities statement

The Trustees (who are also directors of The Myotubular Trust for the purposes of company law) are responsible for preparing the Trustees' Report and the financial statements in accordance with applicable law and United Kingdom Accounting Standards.

Company law in England and Wales requires the Trustees to prepare financial statements for each financial year in accordance with United Kingdom Generally Accepted Accounting Practice (United Kingdom Accounting Standards and applicable law). The financial statements are required by law to give a true and fair view of the state of affairs of the charity and of the income and expenditure of the charity for that period. In preparing these financial statements the trustees are required to:

- Select suitable accounting policies and then apply them consistently
- Observe the methods and principles of the Charities SORP
- Make judgments and estimates that are reasonable and prudent
- State whether applicable UK accounting standards have been followed and statements of recommended practice, subject to any departures disclosed and explained in the financial statements and
- Prepare the financial statements on the going concern basis unless it is inappropriate to presume that the charity will continue to operate.

The trustees are responsible for keeping accounting records, which disclose with reasonable accuracy at any time the financial position of the charity and enable them to ensure that the financial statements comply with the Companies Act 2006. They are responsible for safeguarding the assets of the charity and hence for taking reasonable steps for the prevention and detection of fraud and other irregularities.

Risk management

The trustees have examined the strategic, business and operational risks, which the charity faces and confirm that systems have been established to enable regular reports to be produced so that the necessary steps can be taken to lessen the risks.

INDEPENDENT EXAMINER'S REPORT FOR THE YEAR ENDED 31 DECEMBER 2021

Respective responsibilities of trustees and examiner

The trustees (who are also the directors of the company for the purpose of company law) are responsible for the preparation of the accounts. The charity's trustees consider that an audit is not required for this year under section 144 of the Charities Act 2011 (the Charities Act) and that an independent examination is needed.

Having been satisfied that the charity is not subject to audit under company law and is eligible for independent examination it is my responsibility to:

- examine the accounts under section 145 of the Charities Act,
- to follow the procedures laid down in the General Directions given by the Charity Commission (under section 145(5)(b) of the Charities Act, and
- to state whether particular matters have come to my attention.

Basis of independent examiner's statement

My examination was carried out in accordance with the General Directions given by the Charity Commission. An examination includes a review of the accounting records kept by the charity and a comparison of the accounts presented with those records. It also includes consideration of any unusual items or disclosures in the accounts, and the seeking of explanations from you as trustees concerning any such matters. The procedures undertaken do not provide all the evidence that would be required in an audit, and consequently, no opinion is given as to whether the accounts present a 'true and fair view' and the report is limited to those matters set out in the statement below.

Independent examiner's statement

In connection with my examination, no matter has come to my attention:

- 1) which gives me reasonable cause to believe that in, any material respect, the requirements:
 - to keep accounting records in accordance with section 130 of the Charities Act; and
 - to prepare accounts which accord with the accounting records and to comply with the accounting requirements of the Companies Act 2006 and with the methods and principles of the Statement of Recommended Practice applicable to charities preparing their accounts in accordance with the Financial Reporting Standard applicable in the UK and Republic of Ireland (FRS 102) (effective 1 January 2015) have not been met; or
- 2) to which, in my opinion, attention should be drawn in order to enable a proper understanding of the accounts to be reached



Michael Stone MA ACA
Jamieson Stone LLP
Windsor House
40/41 Great Castle Street
London
W1W 8LU

16 September 2022

STATEMENT OF FINANCIAL ACTIVITIES FOR THE YEAR ENDED 31 DECEMBER 2021

	Unrestricted Funds £	Designated Funds £	Restricted Funds £	Total Funds 2021 £	Total Funds 2020 £
<u>INCOMING RESOURCES</u>					
Donations and grants	58,503	42,500	-	101,003	110,628
Fundraising activities	43,028	-	-	43,028	20,635
Interest received	739	-	-	739	1,602
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Total incoming resources	102,270	42,500	-	144,770	132,865
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<u>RESOURCES EXPENDED</u>					
Research	-	41,289	-	41,289	133,306
Fundraising costs	464	-	-	464	345
Outreach work	-	2,500	-	2,500	-
Staff costs	49,961	-	-	49,961	50,765
Training	719	-	-	719	-
Advocacy	-	13,000	-	13,000	12,187
Family Conference costs	150	-	-	150	-
Accountancy & payroll	140	-	-	140	110
Independent examiner	1,000	-	-	1,000	1,000
Legal & professional	445	-	-	445	-
Trustee's indemnity	873	-	-	873	966
Travel costs	-	-	-	-	216
Printing & stationery	89	-	-	89	72
Postage & telephone	11	-	-	11	56
Subscriptions	174	-	-	174	260
Web & IT costs	1,497	-	-	1,497	688
Bank charges	214	-	-	214	300
Miscellaneous costs	104	-	-	104	-
	-----	-----	-----	-----	-----
Total resources expended	55,841	56,789	-	112,630	200,271
	-----	-----	-----	-----	-----
Net incoming/(outgoing) resources	46,429	(14,289)	-	32,140	(67,406)
Transfers between funds	(45,690)	45,690	-	-	-
	-----	-----	-----	-----	-----
Net movement in funds	739	31,401	-	32,140	(67,406)
	-----	-----	-----	-----	-----
Total funds brought forward	5,962	289,041	-	295,003	362,409
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Total funds carried forward	6,701	320,442	-	327,143	295,003
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All gains and losses arising in the year are included within the statement of financial activities

The accompanying notes form part of these financial statements

Myotubular Trust

BALANCE SHEET AS AT 31 DECEMBER 2021

	£	2021 £	£	2020 £
CURRENT ASSETS				
Debtors and prepayments	25,419		26,275	
Cash at bank and in hand	303,447		270,455	
	-----		-----	
Total current assets	328,866		296,730	
	-----		-----	
CREDITORS				
Amounts falling due within one year	1,723		1,727	
	-----		-----	
Net current assets		327,143		295,003
		-----		-----
NET ASSETS		327,143		295,003
		-----		-----
FUNDS OF THE CHARITY				
Restricted funds		-		-
Unrestricted general funds		6,701		5,962
Unrestricted designated funds		320,442		289,041
		-----		-----
		327,143		295,003
		-----		-----

The company is entitled to exemption from audit under Section 477 of the Companies Act 2006 for the year ended 31 December 2021 and the members have not required the company to obtain an audit of its financial statements for the year ended 31 December 2021 in accordance with Section 476 of the Companies Act 2006.

The directors acknowledge their responsibilities for: (a) ensuring that the company keeps accounting records which comply with Sections 386 and 387 of the Companies Act 2006 and (b) preparing financial statements which give a true and fair view of the state of affairs of the company as at the end of each financial year and of its profit or loss for each financial year in accordance with the requirements of Sections 394 and 395 and which otherwise comply with the requirements of the Companies Act 2006 relating to financial statements, so far as applicable to the company.

The financial statements have been prepared in accordance with the provisions of Part 15 of the Companies Act 2006 relating to small companies.

The financial statements were approved by the trustees on and signed on their behalf:



.....
Andrew Lennox
Chairman/Trustee



.....
James Rosling
Treasurer/Trustee

The accompanying notes form part of these financial statements

NOTES TO THE FINANCIAL STATEMENTS FOR THE YEAR ENDED 31 DECEMBER 2021

1. BASIS OF PREPARATION

The financial statements have been prepared on the accruals basis of historic cost in accordance with the Statement of Recommended Practice applicable to charities preparing their accounts in accordance with the Financial Reporting Standard applicable in the UK and Republic of Ireland (FRS 102) (effective 1 January 2015) and the Companies Act 2006 and the Charities Act 2011.

2. ACCOUNTING POLICIES

Incoming resources

Incoming resources are recognised when the charity becomes entitled to the resources, the trustees are virtually certain they will receive the resources, and the monetary value can be measured with sufficient reliability.

Where incoming resources have related expenditure (as with fundraising or contract income) the incoming resources and related expenditure are reported gross in the Statement of Financial Activities.

Grants and donations are recognised when the charity has unconditional entitlement to the resources.

Incoming resources from tax reclaims are recognised at the same time as the gift to which they relate.

Contractual income and performance related grants are recognized once the related goods or services have been delivered.

Investment income is included in the accounts when receivable.

Resources expended

Resources expended are recognised as soon as there is legal or constructive obligation committing the charity to pay out resources.

Resources expended include attributable VAT which cannot be recovered.

Funds

Restricted funds are funds to be used for specific purposes as declared by the donor.

Designated funds are monies set aside from general funds and designated for specific research awards by the trustees.

Unrestricted funds are donations and all other incoming resources without a specified purpose and which are available as general funds.

NOTES TO THE FINANCIAL STATEMENTS (CONTINUED)

FOR THE YEAR ENDED 31 DECEMBER 2021

3. TAXATION

The Myotubular Trust is a registered charity and is not liable to Income Tax or Corporation Tax on income derived from its charitable activities.

4. RESOURCES EXPENDED

	2021 £	2020 £
Research	41,289	133,306
Independent examiner's remuneration	1,000	1,000
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The principal research grants awarded during the year were as follows:

	2021 £	2020 £
Hnia at Inserm	29,059	-
Tamoxifen Protocol	2,469	-
Newcastle University re Treat NMD	7,500	-
Research Consultancy	2,261	-
Sick Kids Canada - Dr Dowling	-	72,000
Sparks Charity GOSH	-	58,108
Taurus Tide	-	3,198
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5. DEBTORS AND PREPAYMENTS

	2021 £	2020 £
Gift Aid	419	133
Prepayments and accrued income	25,000	26,142
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	25,419	26,275
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6. CREDITORS FALLING DUE WITHIN ONE YEAR

	2021 £	2020 £
Accruals	1,723	1,727
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NOTES TO THE FINANCIAL STATEMENTS (CONTINUED) FOR THE YEAR ENDED 31 DECEMBER 2021

7. RELATED PARTY TRANSACTIONS

There were no related party transactions by the trustees for the year (2020: None).

One Trustee received remuneration of £20,000 during the year ending 31 December 2021 (2020: £20,000).

The trustee's remuneration and other administration costs are funded from unrestricted corporate and charitable donations, which were specifically raised for that purpose.